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Making more AAV

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Abstract

Adeno-associated virus (AAV) is one of, if not the top clinical gene therapy vector in use today. This is because of its high safety record and its ability to transfer genes in such a manner that they are expressed very long term. AAV is described as a "helper-dependent parvovirus" and it requires that AAV receive helper gene functions from a second virus, usually adenovirus (Ad), to replicate at high levels in cells. However, we have also been studying AAV interaction with human papillomavirus (HPV) which also provides helper activity for AAV. Upon looking at the helper genes of both Ad and HPV we find there are dissimilarities, that is, the helper gene sets do not recapitulate each other. For example HPV E1 is a helicase and there is no helicase among the Ad helper gene set. This uniqueness of helper gene sets suggests that combinations of helper genes from Ad and HPV might be used together to give a higher level of helper function and a higher level of recombinant (r) AAV and wtAAV production. Moreover, specific cellular genes are known to be required for AAV replication/production. These cellular genes might also be provided at higher levels to further boost rAAV and wtAAV production. These studies together suggest that there are many opportunities for better understanding AAV molecular biology and expanding AAV's use as a gene therapy vector through higher rAAV yield. These and other topics will be discussed.

Biography

Paul L. Hermonat completed his Ph.D. at the University of Florida in 1984, and postdoctoral studies at the National Institutes of Health. He is a Professor of Internal Medicine and OB/GYN at the University of Arkansas for Medical Sciences and Research Career Scientist at the Central Arkansas Veterans Healthcare System. He mapped AAV's genes, determined their functions, was the first to generate recombinant adeno-assocociated virus (AAV/NeoR) and to transfer genes into cells via this method, and has over 140 manuscripts published. From his first study on AAV-based gene therapy in 1984 there are now over 2,300 papers on the topic.